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| APPLICATION NO. | FILING DATE | FIRST NAMED INVENTOR | ATTORNEY DOCKET NO. | CONFIRMATION NO. |
|---|---|---|---|---|
| 09/851,494 | 05/08/2001 | Ehud Goldin | 3394/1H557US1 | 2229 |

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| EXAMINER | |
|---|---|
| ULM, JOHN D | |
| **ART UNIT** | **PAPER NUMBER** |
| 1646 | |

DATE MAILED: 03/23/2005

Please find below and/or attached an Office communication concerning this application or proceeding.

| Office Action Summary | Application No. | Applicant(s) | |
|---|---|---|---|
| | 09/851,494 | GOLDIN ET AL. | |
| | Examiner | Art Unit | |
| | John D. Ulm | 1646 | |

*-- The MAILING DATE of this communication appears on the cover sheet with the correspondence address --*

**Period for Reply**

A SHORTENED STATUTORY PERIOD FOR REPLY IS SET TO EXPIRE 3 MONTH(S) FROM THE MAILING DATE OF THIS COMMUNICATION.

- Extensions of time may be available under the provisions of 37 CFR 1.136(a). In no event, however, may a reply be timely filed after SIX (6) MONTHS from the mailing date of this communication.
- If the period for reply specified above is less than thirty (30) days, a reply within the statutory minimum of thirty (30) days will be considered timely.
- If NO period for reply is specified above, the maximum statutory period will apply and will expire SIX (6) MONTHS from the mailing date of this communication.
- Failure to reply within the set or extended period for reply will, by statute, cause the application to become ABANDONED (35 U.S.C. § 133). Any reply received by the Office later than three months after the mailing date of this communication, even if timely filed, may reduce any earned patent term adjustment. See 37 CFR 1.704(b).

**Status**

1)☒ Responsive to communication(s) filed on *17 February 2005*.

2a)☐ This action is **FINAL**. 2b)☒ This action is non-final.

3)☐ Since this application is in condition for allowance except for formal matters, prosecution as to the merits is closed in accordance with the practice under *Ex parte Quayle*, 1935 C.D. 11, 453 O.G. 213.

**Disposition of Claims**

4)☒ Claim(s) *1-7,12-27,33-35 and 39-58* is/are pending in the application.

4a) Of the above claim(s) _____ is/are withdrawn from consideration.

5)☒ Claim(s) *1,5-7,33,34 and 39* is/are allowed.

6)☒ Claim(s) *2-4 12-27 35 40-58* is/are rejected.

7)☐ Claim(s) _____ is/are objected to.

8)☐ Claim(s) _____ are subject to restriction and/or election requirement.

**Application Papers**

9)☐ The specification is objected to by the Examiner.

10)☐ The drawing(s) filed on _____ is/are: a)☐ accepted or b)☐ objected to by the Examiner.

Applicant may not request that any objection to the drawing(s) be held in abeyance. See 37 CFR 1.85(a).

Replacement drawing sheet(s) including the correction is required if the drawing(s) is objected to. See 37 CFR 1.121(d).

11)☐ The oath or declaration is objected to by the Examiner. Note the attached Office Action or form PTO-152.

**Priority under 35 U.S.C. § 119**

12)☐ Acknowledgment is made of a claim for foreign priority under 35 U.S.C. § 119(a)-(d) or (f).

a)☐ All b)☐ Some * c)☐ None of:

1.☐ Certified copies of the priority documents have been received.

2.☐ Certified copies of the priority documents have been received in Application No. _____.

3.☐ Copies of the certified copies of the priority documents have been received in this National Stage application from the International Bureau (PCT Rule 17.2(a)).

* See the attached detailed Office action for a list of the certified copies not received.

**Attachment(s)**

1) ☐ Notice of References Cited (PTO-892)
2) ☐ Notice of Draftsperson's Patent Drawing Review (PTO-948)
3) ☐ Information Disclosure Statement(s) (PTO-1449 or PTO/SB/08) Paper No(s)/Mail Date _____.
4) ☐ Interview Summary (PTO-413) Paper No(s)/Mail Date. _____ .
5) ☐ Notice of Informal Patent Application (PTO-152)
6) ☐ Other: _____.

1) Claims 1 to 7, 12 to 27, 33 to 35 and 39 to 58 are pending in the instant application. Claims 2 to 5, 12 to 14, 16 to 18, 20 to 25, 27, 33 and 34 have been amended and claims 40 to 58 have been added as requested by Applicant in the correspondence filed 10 January of 2005.

2) A request for continued examination under 37 CFR 1.114, including the fee set forth in 37 CFR 1.17(e), was filed in this application after final rejection. Since this application is eligible for continued examination under 37 CFR 1.114, and the fee set forth in 37 CFR 1.17(e) has been timely paid, the finality of the previous Office action has been withdrawn pursuant to 37 CFR 1.114. Applicant's submission filed on 10 January of 2005 has been entered.

3) Any objection or rejection of record that is not expressly repeated in this action has been overcome by Applicant's response and withdrawn.

4) The text of those sections of Title 35, U.S. Code not included in this action can be found in a prior Office action.

5) The declaration filed on 10 January of 2005 under 37 CFR 1.131 is sufficient to overcome the Curtis et al. and Lai et al. references.

6) Claims 1, 5 to 7, 33, 34 and 39 are allowable as written.

7) Claims 2 to 4 and 40 to 46 are rejected under 35 U.S.C. 112, first paragraph, as failing to comply with the enablement requirement. These claims contain subject matter which was not described in the specification in such a way as to enable one skilled in the art to which it pertains, or with which it is most nearly connected, to make and/or use the invention. These claims require an "isolated nucleic acid" that "is a

gene". The biological sciences regard a gene as an indivisible unit of inheritance, located in the genome of an organism or virus. Therefore, the terms "isolated nucleic acid " and "gene" are each regarded as mutually exclusive of the other. These claims are not enabled because the instant specification does not provide the guidance needed to make an isolated nucleic acid that "is a gene".

8) Claim 35 is rejected under 35 U.S.C. 112, first paragraph, as failing to comply with the enablement requirement. This claim encompasses subject matter which was not described in the specification in such a way as to enable one skilled in the art to which it pertains, or with which it is most nearly connected, to make and/or use the invention. This claim is drawn to a "pharmaceutical composition" comprising an expression vector encoding a protein of the instant invention. The only method disclosed in the instant specification of using the claimed pharmaceutical composition is presented in the text in lines 24 to 27 on page 3 therein, which states that "another embodiment of the invention provides vectors that express functional human MCOLNI in human target cells and a method of treating a mucolipidosis associated with such a defect by administering the vector into cells (such as bone marrow cells) of the subject" and that "[p]harmaceutical compositions comprising the vector are also provided". It is noted that neither the instant specification nor the art of record describes even a single example of the successful employment of an expression vector in the treatment of a genetic defect. The specific defects associated with a plurality of genetic diseases, including cystic fibrosis and sickle cell anemia, have been well characterized long before the making of the instant invention, and yet the treatment of these disorders via

gene therapy has yet to be realized. Clearly, gene therapy has not reached the status of a routine practice in the art and the instant specification fails to provide the guidance that is lacking from the prior art. A patent is granted for a completed invention, not the general suggestion of an idea and how that idea might be developed into the claimed invention. In the decision of *Genentec, Inc, v. Novo Nordisk*, 42 USPQ 2d 100,(CAFC 1997), the court held that:

> "[p]atent protection is granted in return for an enabling disclosure of an invention, not for vague intimations of general ideas that may or may not be workable" and that "[t]ossing out the mere germ of an idea does not constitute enabling disclosure". The court further stated that "when there is no disclosure of any specific starting material or of any of the conditions under which a process is to be carried out, undue experimentation is required; there is a failure to meet the enablement requirements that cannot be rectified by asserting that all the disclosure related to the process is within the skill of the art", "[i]t is the specification, not the knowledge of one skilled in the art, that must supply the novel aspects of an invention in order to constitute adequate enablement".

The instant specification is not enabling because one can not following the guidance presented therein and employ the claimed pharmaceutical composition in the manner disclosed in the instant specification without first making a substantial inventive contribution.

9) Claims 2 to 4, 12 to 27 and 40 to 58 are rejected under 35 U.S.C. 112, second paragraph, as being indefinite for failing to particularly point out and distinctly claim the subject matter which applicant regards as the invention.

9.1) Claims 2 to 4, 12 to 27 and 40 to 58 are rejected because the limitation "mutation" requires a specific point of reference and none is given. The term "mutation"

is a term that is defined in the art as an inheritable change from a previous form. Therefore, this limitation requires a single point of reference to have any meaning, and the instant claims fail to recite a single point of reference from which the "mutation" has arisen. Claims 3, 14, 18, 22 and 27 are included in this rejection because the sequence identifiers recited therein are contained within parenthesis, making it unclear if they are limiting or only exemplary.

9.2) Claims 23 to 27 are incomplete because they are kit claims that only recite a single component whereas a "kit", by definition, is a collection of two or more items intended to be used together. These claims recite an insufficient number of elements to define a "kit".

9.3) Claims 12 to 22 and 47 to 58 provide for the use of an oliogonucleotide for detecting a mutation, but, since the claim does not set forth any steps involved in the method/process, it is unclear what method/process applicant is intending to encompass. A claim is indefinite where it merely recites an intended use without any active, positive steps delimiting how this use is actually practiced.

35 U.S.C. 101 reads as follows:

> Whoever invents or discovers any new and useful process, machine, manufacture, or composition of matter, or any new and useful improvement thereof, may obtain a patent therefor, subject to the conditions and requirements of this title.

10) Claims12 to 19 and 47 to 52 are rejected under 35 U.S.C. 101 because the claimed recitation of a use, without setting forth any steps involved in the process, results in an improper definition of a process, i.e., results in a claim which is not a proper process claim under 35 U.S.C. 101. See for example *Ex parte Dunki*, 153

USPQ 678 (Bd.App. 1967) and *Clinical Products, Ltd.* v. *Brenner*, 255 F. Supp. 131, 149 USPQ 475 (D.D.C. 1966).

11) Applicant's arguments filed 10 January of 2005 have been fully considered but they are not persuasive.

Any inquiry concerning this communication or earlier communications from the examiner should be directed to John D. Ulm whose telephone number is (571) 272-0880. The examiner can normally be reached on 9:00AM to 5:30PM.

If attempts to reach the examiner by telephone are unsuccessful, the examiner's supervisor, Anthony Caputa can be reached on (571) 272-0829. The fax phone number for the organization where this application or proceeding is assigned is 703-872-9306.

Information regarding the status of an application may be obtained from the Patent Application Information Retrieval (PAIR) system. Status information for published applications may be obtained from either Private PAIR or Public PAIR. Status information for unpublished applications is available through Private PAIR only. For more information about the PAIR system, see http://pair-direct.uspto.gov. Should you have questions on access to the Private PAIR system, contact the Electronic Business Center (EBC) at 866-217-9197 (toll-free).

JOHN ULM
PRIMARY EXAMINER
GROUP 1600